Spotlight on Muscular Dystrophy and Stem Cell Research - Helen Blau, Stanford

Duchenne muscular dystrophy (DMD) is the most severe form of muscular dystrophy that affects 1 in 3500 boys and leads to progressive muscle degeneration and death by the second decade of life. It's been nearly thirty years ago that dystrophin, the mutated gene that causes DMD, was identified. Without the large structural protein encoded by dystrophin, the muscle cell walls become stressed, leaky and eventually degenerate. A mouse model of DMD with a naturally occurring mutation in the dystrophin gene has been available nearly 1989. A lot of therapies have been tested in this model and then tried in humans and not worked.

In this video Dr. Helen Blau, a CIRM grantee from Stanford University, reports that her lab has generated a new mouse model that more accurately mimics the Duchenne symptoms observed in humans. This breakthrough opens up opportunities to better understand the disease and to develop novel therapies.